Putting a Human Face on Biotechnology:

A Report on the Joint Economic Committee's Biotechnology Summit

October 1999

Joint Economic Committee Staff Report
Office of the Chairman, Connie Mack

PUTTING A HUMAN FACE ON BIOTECHNOLOGY:
A REPORT ON THE JOINT ECONOMIC COMMITTEE'S BIOTECHNOLOGY SUMMIT

TABLE OF CONTENTS

OVERVIEW

The growth of America's biotechnology industry has resulted from a remarkable combination of entrepreneurship, innovative capital markets, and federal research investments. The United States leads the world in biotechnology, which is contributing to our strong economic growth and creating substantial improvements in our quality of life.

With the path-breaking success of biotechnology in mind, the Joint Economic Committee (JEC), chaired by Senator Connie Mack (R-FL), held a one-day Summit on September 29, 1999 which focused on "Putting a Human Face on Biotechnology." The Summit heard from over two dozen witnesses including industry experts, doctors, and patients who are benefiting from biotechnology medicines.[1] This report provides a brief overview of the policy issues raised during the Summit, and summarizes the benefits to be gained from biotechnology research, as discussed by Summit participants.

The biotechnology industry offers immense potential for cures to many diseases as it takes advantage of rapid gains in scientists' understanding of human genomics. About 80 biotechnology drugs and vaccines are already on the market and have helped millions of patients. Hundreds of additional products are being researched, or are currently in clinical trials. Biotech medicines approved for use include products to treat anemia, cystic fibrosis, hemophilia, cancer, and other diseases. In agriculture, biotechnology research is leading to greater yields of higher quality crops at lower costs.

These advances have been made possible by the twin strengths of federally-sponsored medical research carried out by the National Institutes of Health (NIH) and other agencies, and the entrepreneurial leadership of about 1,300 U.S. biotech companies. In 1998, the industry generated revenues of about $19 billion, spent $10 billion on R&D, and employed about 150,000 highly-skilled workers. Most biotech companies are fairly small, with two-thirds of firms having fewer than 135 employees.

The biotech industry survives on an inflow of funds from venture capitalists and proceeds from public share issues. These investment inflows are crucial because most biotech firms do not have substantial revenues, and the industry as a whole reports a net loss. Biotech investors often wait years to receive investment returns since it typically takes over seven years and $200-$350 million to bring a new biotech drug to market. Therefore, the encouragement of risky and long-term capital inflows from investors is important to the continued health of the industry – and to the health of people everywhere who benefit from biotech treatments.

The Summit offered Members of the Joint Economic Committee a chance to hear from witnesses about the fascinating progress being made in biotechnology, and about policies that

[1] In addition to hearings, the Summit included a trade fair which allowed biotechnology and medical device companies to demonstrate their leading-edge products.

will promote further growth in the industry. Witnesses focused on the important role that federal research funding plays, but also warned that the industry is very sensitive to adverse federal policy changes, such as price regulations on pharmaceutical products.

This study summarizes the remarks of witnesses at the Summit with regard to biotechnology advances, medical experiences, and policy issues raised. A report section covers each of the four witness panels at the hearings. The Appendix includes a full list of Summit witnesses.

PANEL 1 – SURVIVING DISEASE AND IMPROVING LIVES

Background

Biotechnology has enormous potential to discover new medical treatments that not only improve the quality of life of those with disease, but also save lives. Biotech research is already playing an important role in the fight against many diseases. For example, the biotech industry is creating a new class of drugs that can alleviate the devastating effects of cancer. Herceptin, a biotech drug created by Genentech, is offering new hope to breast cancer patients. The great hopes for further advances by the industry were expressed by Senator Mack when he stated that, "many of us believe that the biotech industry will be to the 21st century what computers were to the 20th century."[2]

Federal investments in basic medical research, sponsored primarily by the National Institutes of Health (NIH), have played a major role in the success of the biotech industry. Entrepreneurial biotech companies are harnessing the research advances made from NIH funding, and are bringing breakthrough medical products to the market. The importance of NIH funding has prompted Senator Mack and other Congressional leaders to call for a doubling of the agency's budget over the next five years. In his opening statement at the Summit, Senator Mack noted:

> I am strongly committed to doubling funding for the NIH over the next five years. Medical research is the key to saving lives. We are on the verge of discovery in so many different areas of disease. It is crucial we provide our scientists with the tools necessary to continue the tremendous advances being made in biomedical research. My wife is alive today, my daughter is alive today, and I am alive today because of the many advances made in cancer research and early detection.

Discussion

Witnesses in the Summit's first panel included patients and doctors who described some encouraging success stories resulting from biotechnology and medical advances. In addition to describing their stories and ground-breaking treatments, witnesses highlighted the strength of human determination to overcome illness. As Senator Mack stated, cancer survivors and patients of other life-threatening diseases are a testament to the human spirit and the will to survive.

Lance Armstrong, winner of the 1999 Tour de France cycling race and a testicular cancer survivor, stated that he is a living example of the benefits of cancer research investment. In his testimony, Mr. Armstrong emphasized the important message of early

[2] Senator Mack interview with Deborah Marchini, CNNfn anchor, Business Day, September 29, 1999.

cancer detection. Ignoring the initial signs of cancer before eventually seeing a doctor, he knew that he was very lucky to have received such successful treatment at a late stage. He told the Summit that:

> If we can get the message out and teach people what to look for and hopefully provide them with living examples of a cure and life after cancer, then I think that they won't be so hesitant to go to the doctor that first time and get that check-up. These aren't pleasant ... But we're talking about life and death here.

Mr. Armstrong's physician, **Dr. Larry Einhorn**, told the Summit that prior to modern research, testicular cancer was generally a death sentence. Currently, testicular cancer is the most prevalent form of cancer in men aged 15 to 35. Dr. Einhorn said Armstrong would not have won the Tour de France if he had taken the standard drug for cancer treatment, which has the unfortunate side-effect of reducing a patient's lung capacity. Instead, he took Ifosfamide, an alternative treatment offered by Dr. Einhorn**.** Although this drug is more likely to cause nausea and illness in a patient, it is less likely to damage a patient's lungs. By successful use of this drug, Armstrong was able to continue his cycling career, which ultimately culminated in his recent victory in the Tour de France.

The Summit also heard from **Erin Fagan**, a kidney transplant recipient. She described how she became anemic as a dialysis patient. Due to eventual failure in her transplanted kidney, she lacked the hormone which maintains a proper level of red blood cells to carry oxygen to the rest of the body. As a result, Ms. Fagan suffered from debilitating fatigue and weakness, thus substantially reducing her ability to lead a productive life.

In the past, patients such as Ms. Fagan would undergo repeated and potentially dangerous blood transfusions, and suffer the unwanted side-effects of standard androgen therapy. Ms. Fagan opted to undergo dialysis and receive Epogen to replenish oxygen-carrying red blood cells. Epogen is produced by a leading biotech company, Amgen, and is giving patients an important new treatment option.

Epogen is essentially a copy of the natural hormone that corrects the level of red blood cells. Erin Fagan's physician, **Dr. Julia Breyer-Lewis**, told the Summit:

> Epogen is actually, I think, a wonderful success story. Erythropoietin is a hormone made by the kidney that makes you make blood in the bone marrow. So it gets transported in your blood from the kidney to the bone marrow. It's a wonderful story. The NIH funded basic research in the 1970s, where Epogen was purified from human urine. It was classic laborious protein biochemistry. No one at the time could foresee where it would go. As a result of Epogen, Ms. Fagan regained her strength and currently is able to work a full-time job. Because of the basic research

> by the National Institutes of Health, plus the innovative efforts of biotechnology, people such as Erin Fagan gain a new vitality in life.

Another biotechnology success story is **Carolyn Boyer-Fortier**, a breast cancer survivor who was treated with the drug Herceptin. Diagnosed in 1993 with an advanced case of cancer, Ms. Boyer-Fortier underwent aggressive chemotherapy which caused her extreme sickness. While the chemotherapy apparently cleared her of the cancer, in 1996 it was discovered that the cancer had spread to her liver as a result of metastatic disease. Unfortunately, there is no known cure for metastatic breast cancer. At that point, Ms. Boyer-Fortier volunteered for a clinical trial to take Herceptin, a drug developed by Genentech. Patients call Herceptin a “smart bomb” drug because it targets the cancer cells, thus avoiding toxicity to the rest of the body. Ms. Boyer-Fortier told the Summit:

> I hope that "smart bomb" anti-cancer drugs such as Herceptin will take cancer patients away from the cold brutality of chemotherapy treatment and move us towards a treatment that specially targets the cancer without targeting the whole person.

Dr. Steven Shak, a senior director at Genentech, called Herceptin one of the jewels of biotechnology. He told the Summit that unlike some pharmaceutical products which can contain high levels of toxins, Genentech creates drugs based on human proteins which are better tolerated biologically. Addressing the role of federal policy, Dr. Shak noted that without the research and development (R&D) tax credit, the project that produced Herceptin may not have happened at Genentech. Dr. Shak stated:

> The success of Herceptin, I believe, signals an end of the beginning in our fight against breast cancer. We can understand what makes a cancer cell grow uncontrollably, and design with biotechnology new drugs that target the cancer. I should stress that without the R&D tax credit, it's very likely this incredible success story - a story which marks the beginning of a new era of treatment for all kinds of cancer - would not have occurred ... In short, this is a project that was "on the bubble." For projects "on the bubble," the additional revenue made available by the existence of the R&D tax credit is the difference between termination and completion. In this instance, it was the difference between termination and an advance of tremendous significance to patients.

To encourage companies to put additional resources into research, Senator Mack has introduced legislation to make the R&D tax credit permanent. Senator Bill Frist (R-TN), a former heart surgeon, also stressed the importance of medical industry R&D in his comments at the Summit. He noted that the U.S. biotech industry spent an enormous $10 billion on R&D in 1998. Senator Mack believes that extending the R&D tax credit is a good way to

give biotechnology companies the incentives needed to continue making research breakthroughs.

In addition to a strong federal research commitment, the freedom to explore without excessive regulation is important to continued growth in the biotechnology industry. On this point, Senator Robert Bennett (R-UT) noted:

> [T]he most important thing is the attitude and atmosphere of the researchers to explore virtually anything and go virtually any place ... one of the most damaging things to research is when some kind of outside agenda is imposed on the researchers.

Impressed by the testimony of the witnesses on the first Summit panel, Senator Jeff Sessions (R-AL) commended the valuable contributions of the physicians and patients. He stated:

> [I]t does help us as a nation, as part of our strength as a nation, and we are benefiting the entire world in an extraordinary way by the research that so many of you have been involved in.

In conclusion to the first panel, **Dr. Arthur Ullian**, Chairman of the Task Force on Science, Healthcare and the Economy, emphasized to the Summit the important interactions that the health care industry has with the broader "knowledge economy:"

> Not only does the health care delivery system contribute to [economic] growth itself, but the knowledge it has generated in genomics and cell biology is rippling out to other sectors and other industries in ways that we're just beginning to see ... Other industries, such as plastics, electronics and computer industries, are finding whole new markets for expansion in health care and health-related research ... The result is the creation of new sciences, new products and new economic activity at a huge scale ... And unlike an asset and resource based economy, which depreciates and depletes itself over time, a knowledge-based economy actually appreciates and builds on itself in an increasingly rapid pace.

PANEL 2 – A COMMITMENT TO RESEARCH

Background

Look in any medicine cabinet in the country and you're likely to find the results of some remarkable medical breakthroughs. More and more of these medical breakthroughs are coming from America's biotechnology companies which are creating an invaluable contribution to the nation's health care system. Biotech innovations include new prescription medications and advanced therapeutics which cure disease, ease symptoms, alleviate pain, and improve the quality of life for millions.

The U.S. biotechnology industry is by far the largest in the world and continues to develop rapidly from a beginning just 25 years ago. The discovery of recombinant DNA and monoclonal antibody technologies in a lab in the 1970s marked the birth of biotechnology. The industry's growth has been built on learning how to apply these basic findings, and the creation of an industry structure which is distinct from larger, traditional pharmaceutical companies. In 1998 alone, the Food and Drug Administration (FDA) approved 30 new biotech drugs and nine new biologics, two of which are aimed at combating breast cancer.

The Summit heard from **Henri Termeer**, CEO of biotech firm Genzyme Corporation, who characterized the changes occurring in the industry as "a revolution." The industry is changing the approach to conquering many diseases and chronic conditions. Genomics, which is the study of the human genetic blueprint, is changing the way diseases are understood and treated. High-tech tools are revolutionizing the discovery and development process enabling researchers to automate the process of identifying compounds. Advanced computer and animal models are allowing faster determinations of drug safety and efficacy so as to move forward into human clinical trials more expeditiously. These advances will require adaptation in federal regulatory structures to ensure quick access to new drugs and therapies.

Leaders of biotechnology companies and biomedical researchers on the second Summit panel discussed challenges facing the industry. Some of the issues discussed included the importance of basic research funding, industry R&D incentives, the rapid pace of innovation, the drug regulatory process, and improved access to new therapies.

Discussion

The U.S. biotechnology industry's foundation and continued strength lies in its commitment to research. This commitment was stressed by **Dr. Arthur Levinson**, CEO of Genentech:

> The pharmaceutical industry will typically spend maybe between 8 and 20 percent of its total revenues on R&D. That is actually high by high-tech standards. ... the semiconductor electronic industry ... spend[s] in

> the low single digits on research. Our company over the last few years has spent no less than 30, and as much as 50 percent, of total revenues on research and development. And that's not that atypical of biotechnology. And I think it's very important to understand the extreme expense rate because this is a very new industry and it is critically dependent upon innovative science.

This commitment to research is one of the reasons why the U.S. is the world leader in biotechnology and biomedical research. Mr. Termeer described the U.S. leadership position in biotech research:

> The amount of basic research spending ... outside the United States is a fraction of what it is here. The biotechnology industry in Massachusetts probably spends more by itself than the whole biotechnology industry of significant countries in Europe, like France. So it is a matter of starting from the same base. Indeed, the technology is very available in Europe or in Japan or elsewhere. Then it is a matter of investing in the application of technology. And that is -- we're way ahead infrastructurally and in terms of actually spending the money.

In addition to the industry's own high level of R&D investment, biotechnology advances rely on research by the NIH and the academic researchers and facilities it supports. Dr. Levinson noted this importance of federal support:

> It is not an overstatement to say that without that [federal] funding, there really would be no biotechnology industry.

Dr. Lewis Edelheit, Senior Vice President of General Electric Corporate R&D, credited the combined strength of public and private research funding as the strength of U.S. biotech:

> I think there's a combination here of government funding basic research and then this huge venture capital industry in the United States, which is also somewhat unique in the world, that leads us, our ability in new industries like biotech, to move much faster than the rest of the world.

While the biotechnology industry is creating a constant stream of new products, for patients, medical advances can never be fast enough. While drugs are discovered and developed faster and more efficiently today than ever, the Biotechnology Industry Organization estimates that new biotech products take an average of 7 to 12 years to develop and bring to market. The time frame of the federal regulatory and approval process is

extremely important to both the producing company, and to the patients who are awaiting new products.

The FDA, the main federal agency charged with overseeing the biotechnology industry, has streamlined its review and approval process in recent years. The agency is tasked with keeping up with the rapidly changing technology associated with the production of these new products, while continuing to instill public trust in product safety.

Product safety is an important concern both for the medical biotech industry, and the other main segment of the biotech industry – agriculture. **Hendrik Verfaillie**, President of Monsanto Company, described advances in biotech agriculture at the Summit. Regarding concerns over the safety of genetically modified food, he noted:

> We must work together on several key issues, including constantly refining the regulatory process as the technology grows. This is absolutely critical to maintain public confidence ... Public confidence in the safety, science and oversight of these technologies is absolutely critical. When that confidence is challenged, as we see today in Europe, issues such as trade and suspicion dominate the public discussion.

Rapid advances in biotech research are not only challenging the FDA, but also the Health Care Financing Administration and insurance companies who must adjust to new medical realities, according to some Summit panelists. The U.S. health care industry has a complex system of reimbursement policies that work well for some patients, but not for others. In some situations, reimbursement policies are not sufficiently flexible nor adaptable to innovations in the industry. Mr. Termeer described this problem:

> I do not think that we can fit what's being created into the way that we are doing things. HCFA is a very good institution, well intended when it was set up. I don't think HCFA as an institution can absorb the level of innovation that is occurring.

Dr. Christine Cassel, of Mount Sinai School of Medicine, also discussed the issues of reimbursement and appropriate medical therapies. She noted:

> I think that we really must look at ways not to have people doing the most expensive thing because that's what Medicare covers or in other private insurance, but to do the right thing and the most appropriate thing. We haven't yet really figured out a cost containment strategy that allows us to do that. There are some ideas, but, unfortunately, health policy too often starts out being budget policy rather than starting from the premise that we have these dramatic new advances in medicine and the capability to keep people healthy.

From this discussion, Senator Bennett concluded that Medicare, Medicaid, and insurance companies need to focus more on the needs of the patients, as opposed to what rigid rules say that patients are eligible for, as a means of directing our health care dollars to the right place.

Dr. Levinson best summarized the purpose of the biotechnology industry and underscored the theme of the Summit when he stated:

> All the cutting edge science and innovative technology of our industry is valuable only when it results in the alleviation of human suffering and the overall enhancement of life.

PANEL 3 – FINANCING THE BIOTECH INDUSTRY

Background

Innovative and efficient financial markets have been central to the success of the U.S. biotechnology industry. This is because profits may not be generated for years during research into new products before they are brought to the market. Young biotech firms must therefore look to external financing in order to meet their demands for research funding.

The industry's external financing comes primarily from a mix of private and public equity. Private equity in the form of venture capital has helped hundreds of biotech firms get off the ground. Venture capital firms not only provide equity funding to growing biotech companies, they also assist in product and strategy development, and they recruit experienced managers to guide young firms. Venture capital financing fueled the initial growth of many leading biotech firms, such as Genentech.

U.S. venture capital investment has surged in the past three years from $7.4 billion in 1995 to $25.3 billion in 1998, according to the National Venture Capital Association (NVCA). In 1998, 60 percent of venture capital investment went to information technology and communications firms, 20 percent to non-technology firms, and 20 percent to health and medical firms - of which 6 percent went to biotech firms.

While venture capital assists young biotech firms, initial public equity offerings (IPOs) on NASDAQ have allowed biotech firms to raise substantial amounts of funds for rapid and open-ended future growth. IPOs have raised billions of dollars for U.S. biotech firms, and the high volume of U.S. IPOs has led to increased venture capital funding in anticipation of the future benefits of going public. A *Washington Post* technology columnist recently noted: "to a large extent, the biotech industry is the legacy of NASDAQ ... [B]iotech financing ... is a phenomenon that could only have been produced by the U.S. capital markets, with their diverse and democratized sources of funds."[3]

Discussion

The crucial importance of venture capital to the biotechnology industry was described to the Summit by **Dr. M. Kathy Behrens**, a director of the National Venture Capital Association (NVCA):

> ...without patient [i.e. long-term] investment from venture capitalists, the industry would not exist today. According to NVCA statistics, since

[3] "From Biotech, a History Lesson for Internet Investors," Jerry Knight staff reporter, *Washington Post*, September 6, 1999.

> 1990, venture capitalists have invested over $5.8 billion into over 550 biotechnology companies.

Dr. Behren's views on the industry were particularly useful to the Summit because of her background in securing venture capital for many biotech companies at the firm of Robertson Stephens Investment Management. She also has a doctorate in microbiology, has performed genetic research for six years, and has sat on the boards of many biotech companies. Her experience has allowed her to witness that, "biotechnology is giving new and renewed hope for people who suffer maladies across virtually the entire spectrum of diseases and afflictions."

Despite the remarkable success of the industry, Dr. Behrens thought that there were "serious and profound problems" facing the industry at the current time. She noted:

> While the venture capital industry is investing more money in more companies than ever before, the amount of money going into biotechnology as a percentage of overall dollars disbursed is beginning to decline. While venture capitalists increasingly pursue Internet deals which have the ability to garner a significant financial return in a relatively short time-frame, the biotech arena, which faces heavy government regulation and requires longer-term and more patient investing, naturally suffers. It is likely in 1999 that fewer new biotechnology companies will be funded by venture capitalists. Also, public biotechnology companies are having problems securing additional needed money to get through the FDA pre- market approval process. The market for biotech initial public offerings declined from 22 in 1997 to 6 in 1998, and 1999 does not look very optimistic either.

Dr. Behrens noted that in 1990, about 8 percent of total disbursed venture capital went into biotechnology. In 1999, the comparable figure will be just 3 percent. Also, privately-held biotech companies are having problems securing additional money to get through the FDA drug approval process.

Dr. Behrens also discussed how federal policy affected venture capital funding of biotechnology:

> At this moment, and in this very building, significant debates are taking place regarding health care reform, patent reform, tax policy, immigration policy, and research funding for related fields. The results of these debates may directly affect the future of biotechnology companies and in turn impact the availability of the innovative products these companies are developing.

In particular, Dr. Behrens outlined a number of policy threats which she believed could be damaging to the future funding of the industry. These are:

- Health Care Policy: "Health care proposals which impose drug price controls, or Medicare drug benefits which provide marginal reimbursement, can create a perception or reality that the industry's potential return is limited or at greater risk;"

- Patent Reform: "The biotechnology community has for years been asking for patent reform, only to get bills passed in the House and have them not acted upon in the Senate. The issue is simple -- we need to make certain that the new 20-year GATT patent term does not end up shortening the terms of patents when the government causes delays in the issuance of a patent. Last month, the House passed the American Inventors Protection Act by a vote of 376 to 43. The NVCA hopes that the Senate will act quickly as well;"

- Skilled Work Force: "Advances in biotechnology will only occur with a highly educated workforce. Education reform is critical, but it will not occur overnight. [Therefore] in order to ensure the continued dynamism of the biotechnology industry, Congress needs to increase the number of H1-B visas available. New visas will be available shortly with the commencement of the new fiscal year, but this relief will only be temporary as the number of visas we need to keep our companies running is much larger than the number of visas available in the upcoming year;"

- Accounting Rules: "The biotechnology industry currently is undergoing tremendous consolidation through mergers and acquisitions, which on the whole is positive for the industry. Pooling has been the most desired method of structuring acquisitions, but the Financial Accounting Standards Board has proposed eliminating pooling of interest accounting. By limiting our ability to use mergers and acquisitions as an exit strategy, you may limit our ability to invest in biotechnology. I understand that the Senate Banking Committee is looking at holding hearings on this issue and I would urge you to support this effort so that you can hear at least the reasons why changes in pooling accounting could very well limit the amount of capital invested in biotechnology."

On this panel, the Summit also heard from **Matthew Andresen**, President of Island ECN, Inc. He discussed the role of efficient financial markets in raising funds for leading edge industries, such as biotechnology. Mr. Andresen's company is one of about half a dozen electronic communications networks (ECNs) that are a growing presence in the U.S. securities industry. He noted that ECNs are "a new business model that was made possible by sweeping SEC progressive regulation in January of 1997." Island now trades over 120 million shares a day, accounting for about 12 percent of all the transactions on the NASDAQ exchange.

Island and other ECN's are seeking regulatory approval from the Securities and Exchange Commission (SEC) to become full stock markets. Such advances in electronic trading are making U.S. capital markets more efficient and will make it easier for young high-

tech firms to raise money for expansion. Mr. Andresen described a number of ways in which efficient stock trading can increase capital available to start-up technology companies:

> Island's goal is to create a secondary equity market that is transparent, fair, and efficient. The achievement of this goal will increase investor participation and, in turn, the amount of capital available to fund biotechnology as well as many other types of companies.

In addition, efficient public equity markets can increase private equity funding of high-tech start-ups because venture capital investors anticipate a return on investment from a future public share offering, as Andresen explains:

> Island is making an important contribution toward making venture capital financing more available ... A venture capitalist's key motivation is the possibility of returns far in excess of the risk-free rate of return. Increasingly, the so-called "exit strategy" for a venture capitalist is an Initial Public Offering or IPO. As the [number] of investors willing to participate in the market has increased substantially, we have seen a growing appetite of investors for shares of IPOs. In turn, the success of IPOs encourages venture capital companies to make further investments which creates a "virtuous circle" of investment.

Mr. Andresen also described how the roles of private and public equity investments are changing in today's financial markets:

> Another impact of the increased demand for IPOs is a reduction in the amount of venture capital needed prior to accessing the public market. Traditionally, a private company would go through a number of rounds of venture capital financing until it was deemed ready to go public. Now, companies are going public, in many cases, after just one round of venture financing often eliminating the so-called "mezzanine" financing. In short, the equity markets themselves have become a source of venture capital. Average Americans can now participate in "public" venture capitalism.

Reiterating testimony from Dr. Behrens of the NVCA, Mr. Andresen noted that the huge amount of investment in Internet start-ups in the past few years may have diverted start-up capital away from biotech firms. He noted that capital for the biotech industry raised through IPOs declined 48 percent in 1998. One cause he pointed to for the funding advantage of Internet over biotech firms is that Internet firms "don't need FDA approval or other such government approval [and therefore] can raise public money very early."

Although there are recent signs of improvement, Mr. Andresen thought more needed to be done to ensure the continued growth of the biotechnology sector. He stated:

> Biotechnology is a cash intensive business and its importance in improving all of our lives demands that we find ways to ensure its continued health and vitality. While Island is not an expert on the biotechnology sector specifically, we do believe that one of the best ways to provide biotechnology companies access to capital (whether it be through venture capital or through an IPO) is to ensure that the trend towards cheaper, fairer, more transparent and more accessible capital markets continues.

James Glassman, a Fellow at the American Enterprise Institute, focused his testimony on the importance of increasing savings rates in the United States. He noted that a substantial amount of the capital fueling America's current growth is coming from abroad:

> This is a nation of wonderful entrepreneurs, hard-working scientists, great managers, fabulous ideas. But they can't bring products to the market place without capital. And where does that capital come from? Lately, much of it has been coming from abroad. For the past 17 years, while the U.S. has undergone the single greatest round of prosperity in its history, capital inflows for portfolio investment purchases -- that is, stocks and bonds -- have increased at an average annual rate of 19 percent ... Foreign direct investment has increased at an average annual rate of 15 percent.

Mr. Glassman cautioned that the strong flow of capital from abroad will not necessarily continue unabated. Right now, the U.S. is attracting capital because of our comparatively moderate taxes and regulations, low inflation, and entrepreneurial culture. But he noted that Europe and Asia are making economic reforms and restructuring their businesses. As a result, the rest of the world will become a better place to invest, and foreign capital may not be quite as easy to attract to the United States in the future.

For this reason, Mr. Glassman thought that increasing domestic savings rates is the key to ensuring a long-term supply of U.S. investment capital. He noted that the current U.S. personal savings rate is actually negative by one measure. He discussed some of the causes of the low U.S. savings rate. Aside from a culture of consumption, Mr. Glassman pointed to the federal tax code as a major problem:

> The other reason that Americans aren't saving and investing enough is that public policy tends to deter it. Americans are not investing because we have a tax code that encourages consumption over savings. We tax the earnings of corporations, then we tax the dividends they pay, then we

> tax the income from the dividends and the capital gains that result. And then, as if that weren't enough, we tax the estates of people with the prescience and the discipline to invest for the long term.

In closing, Glassman reiterated that the importance of capital is more than about just economics:

> In the case of the biotechnology industry, capital saves lives. There is no substitute for it and the need is urgent.

Peter Lynch, Vice Chairman of the Fidelity Management and Research Company, discussed another aspect of the financing of high-tech industries - the need for federal support of research and development (R&D). Mr. Lynch thought that the decline in the ratio of federal R&D spending to GDP in the last few decades is creating a "danger zone." He noted:

> ... in rough numbers, [the ratio] peaked in the early '60s at 1.7. It's now down to 0.7 ... I would not want to own a company if it had this kind of a trend line. This is one aspect of [federal] spending that really has a payback. And it has a massive payback in terms of jobs, in terms of taxes paid. Now offsetting that to some degree, but they're mutually exclusive, spending by businesses has gone from 0.7 to 1.7 percent of GDP.

In his testimony, Mr. Lynch stressed the payback received by government and society for federal R&D investment. To illustrate the payback to government he noted:

> And the final element I want to bring is that this year, biotechnology companies alone are going to pay a billion dollars in income taxes -- one billion ... But I think the next two or three years, perhaps the next four years, that number is going to triple. There's about 30 companies that are in the final stages of clinical trials that are going to have products out there. So I think that billion dollars of income taxes is going to go to $3 or $4 billion. So the money that the Federal Government puts out, they get back in products, lower health care costs, healthier consumers, happier citizens, and cash. So as an investor, that's what I like.

Mr. Lynch also noted that Americans need to be made more aware of the important work done by the National Institutes of Health:

> The other point is I think the average person in America thinks the National Institutes of Health is part of the United Way. I don't think they really think it's a line item on the budget. I think they think it's a charity.

They don't call their congressman to say, this is really a good thing. It gets very little credit in the media and the public. I think I'm pretty well read. I didn't know very much about it until three years ago.

PANEL 4 – COST SAVINGS FROM MEDICAL TECHNOLOGY

Background

In Panel 4, witnesses described new biotechnology products, and discussed patient access to coverage for new technologies. Testimony focused on cochlear hearing implants, gene therapy, the rheumatoid arthritis drug Enbrel, new products for blood transfusions, and medical devices to regulate heartbeats and prevent strokes.

Cochlear implants aid people with severe damage to the inner ear. By stimulating nerve fibers with electrodes, the implant device sends a signal from the ear to the brain allowing the person to hear sound. During the past 20 years, more than 12,000 persons worldwide have received cochlear implants. Cochlear implant technology has the potential of helping nearly 1 million hearing-impaired people.

The goal of gene therapy is to supply a patient's cells with healthy copies of missing or flawed genes in order to prevent certain types of cancer and disease. Ultimately, instead of drug therapy to treat or control the symptoms of a genetic disorder, gene therapy may allow physicians to correct disorders by altering the genetic makeup of patient's cells. In gene therapy trials, cells from the blood or bone marrow are removed from the patient and cultivated in a laboratory. A healthy gene is inserted into the cell culture with the help of a disabled virus. The healthy gene alters the patient's cells, a task that the mutated gene could not perform, and the altered cells multiply before being returned to the patient's body.

Rheumatoid arthritis (RA) is a chronic, progressive, and inflammatory disease that afflicts two million Americans. RA occurs when the body’s immune system mistakenly attacks the joints and surrounding soft tissue causing joint inflammation. Biotech firm Immunex has developed a new drug, Enbrel, to fight RA. This drug is a product of recombinant DNA technology, and works by binding to the substance in the body that mistakenly attacks the joints of RA sufferers thus reducing inflammation.

Each year, about four million people in the United States are in need of blood transfusions. Although more than 12 million units of blood are donated each year, regional blood shortages are quite common. The Summit heard testimony from Biopure Corporation, which specializes in developing substitutes for donated blood called “oxygen therapeutics.” These "blood substitutes" are intravenously administered into the circulatory system to deliver oxygen to the body's tissues. The advantages of developing blood substitutes are enormous. Not only would these products be available in the case of a blood shortage, they also have a two-year shelf life and are compatible with all blood types.

The Summit also heard testimony from Guidant Corporation, which created the world’s first implantable defibrillator in 1985. Currently, Guidant Corporation manufactures implantable cardioverter defibrillators which treat rapid heart rhythms, and pacemakers which

speed up slow heart rhythms. Among other products developed by Guidant are new medical instruments which help surgeons perform minimally invasive surgical procedures, thus reducing patient recovery time.

Discussion

The wide range of medical advances occurring in the U.S. health care market were highlighted by the diverse range of witnesses testifying in Panel 4 of the Summit. In a presentation at the beginning of the panel, **Dr. John Niparko** discussed the cochlear implant received by his patient, four-year old **Rachel Noble**. Rachel was diagnosed as being deaf when she was only two weeks old, and was fitted with the implant at 18 months of age. Dr. Niparko explained to the Summit the workings of the cochlear implant:

> The implant is a remarkable blend of digital circuitry and information processing and the device represents an alliance of strategies of processing information that use both manufactured and natural neural circuits, enabling the hearing pathway to respond to sounds of the environment, to voiced sounds, and to provide a very sensitive level of hearing.

Cochlear implants present an interesting example of cost savings possible from advanced medical technology. While implants are expensive, Dr. Niparko thought that its cost and rehabilitation would be less than the yearly attendance costs at a typical school for the deaf. Because of the early age at which Rachel received the implant, she is able to be in a mainstream nursery school. The increased productivity and self-reliance that a hearing impaired individual can achieve with the implant makes the device an excellent investment.

Senator Frist noted that the device was also a good example of why it is important to support basic research in a wide variety of scientific disciplines:

> [I]t's going to be at the overlap of various disciplines that we invest in that the great breakthroughs are going to occur ... it's going to be that integration of engineering, bio-engineering, physics, chemistry, bio-compatibility.

The Summit received testimony via video conferencing by two doctors from Vanderbilt University Medical Center. **Dr. David Carbone** told the Summit how biotech research has helped in the search for cancer therapies. He emphasized how biotech cancer therapies can target diseased cells specifically, thus eliminating the toxicity caused by conventional cancer treatments. **Dr. Joseph Smith** emphasized the importance of collaborations between university researchers and biotech companies. He spoke of the

importance of gene therapy, especially in an area like prostate cancer where conventional therapy has exhausted its potential.

Edward Fritzky, CEO of Immunex Corporation, discussed Enbrel, his firm's biotech drug used for treating rheumatoid arthritis. Mr. Fritzky's testimony captured the excitement that many biotech experts feel about the large potential of the industry. Mr. Fritzky gave strong support to the role played by the NIH, which conducts a great deal of the basic biotech research that is transferred to firms like Immunex.

Mr. Fritzky's testimony highlighted the economics of new biotechnology products. Enbrel treats the painful joint inflammation and soft tissue erosion that makes it difficult for RA sufferers to perform simple tasks like getting out of bed, shaving, or dressing. As a result, he estimated that Enbrel helped reduce the approximately $18 billion a year in lost worker productivity due to RA.

Mr. Fritzky considered the complex question of patient accessibility to new leading-edge treatments. For example, Enbrel is self-injectable and can be administered by the patient at home without a physician. But Medicare recipients, who are the majority of RA patients, are not covered for Enbrel even though it could save the health care system money. Senator Frist recognized this problem and stated that, "a drug like this would keep people out of the hospital. It gives back quality of life. But it really does mean we have to look at where those savings might be by modernizing the overall rigid system."

Carl Rausch, CEO of Biopure Corporation, described to the Summit his firm's development of substitutes for donated blood called "oxygen therapeutics." To date, Biopure has developed and manufactured two hemoglobin-based oxygen therapeutic products. Hemopure is currently being evaluated for human use, and Oxyglobin is an FDA-approved veterinary product for treating anemia in dogs. According to Mr. Rausch, the United States could see a shortage of 300,000 or more donated blood units next year. Mr. Rausch expressed concern that while safety regulations on donated blood have become more stringent during the last 20 years making blood collection more costly, Medicare has not changed its payments to those companies involved in the manufacture of blood-related products.

The availability of a blood substitute such as Hemopure could help alleviate a blood shortage, and can eliminate the risk involved in blood transfusion. Mr. Rausch, stated that, "eight out of 10,000 units of blood pose a potential serious risk to the recipient. Red blood cells have been associated with substantially greater infection, such as pneumonia, and conditions resulting in increased hospitalization costs of approximately $14,000 per patient." Mr. Rausch pointed out that this hidden cost of blood-related expenses drives up the overall cost of health care. These costs could be eliminated with the use of oxygen therapeutics.

Ronald Dollens, CEO of Guidant Corporation, discussed some of the federal policies that affect innovation in the biotech and medical device industries. Mr. Dollens urged policymakers that when considering such issues as the R&D tax credit, to focus on the

potential of medical technology to improve and save lives. He also discussed how medical technology industries were sensitive to developments in Washington:

> If I look at 1993, where the FDA was being nonresponsive and we were talking about a Clinton health care plan that limited people's choice, we saw very little capital formation and very little capital appreciation [in health care companies]. [But in] 1996, the FDA was making some great strides in terms of product approvals. We had 65 initial public offerings that year. In 1999, with concern about Medicare reform and reimbursement, there have been zero IPOs in the medical device sector.

Dr. George Rathmann, Chairman of ICOS Corporation, is considered to be the "godfather" of biotechnology in America, having co-founded Amgen in 1980 and ICOS in 1990. Dr. Rathmann's testimony described why the United States is the world leader in biotechnology research and gave credit to our excellent higher education system and basic research infrastructure largely supported by the federal government. He also gave credit to the free market pricing of biotech products, and noted that the threat of price controls has a negative effect on biotech investment:

> In 1994, with the threat of price controls ... the price of many of our stocks dropped by 80 percent. ICOS dropped by 82 percent during that year. It looked as if we might not be able to get a refinancing. About three or four weeks ago, there was a hearing with respect to the issues of what Medicare changes might mean. Our stock dropped 20 percent in about a week. So the linkage has never been so clear as to the need for a free market with respect to how products will be priced.

Senator Mack echoed some of Dr. Rathmann's themes as he closed the Summit. He stated that:

> [I]n health care in America, we have people who come from all over the world to get the best health care system … 90 percent of biotech products are being produced in America, and that's going to stay that way for a long, long time … in a free market, capitalist system, which I happen to love, we are able to provide this leadership, and we are in fact able to give hope, not just to Americans, but hope to people around the world.

Prepared by JEC staff members Chris Edwards (Overview and Panel 3), Joe Jacquot (Panel 1), Josephine Robinson (Panel 2), and Angie Ritzert (Panel 4). Please contact the JEC (202-224-5171) with any questions or comments.

APPENDIX: SUMMIT WITNESSES

Wednesday, September 29, 1999

Panel 1

Lance Armstrong, winner of the 1999 Tour de France and testicular cancer patient.
Dr. Larry Einhorn, Mr. Armstrong's physician.
Carolyn Boyer-Fortier, breast cancer patient.
Dr. Steven Shak, Senior Director of Medical Affairs, Genentech, Inc.
Erin Fagan, kidney transplant patient.
Dr. Julia Breyer-Lewis, Diabetic Nephrologist.
Joan London, rheumatoid arthritis patient.
Dr. Robert Bunning, Director, Arthritis Program, National Rehabilitation Hospital.
Dr. Arthur Ullian, Chairman, Task Force on Science, Healthcare, and the Economy.

Panel 2

Henri Termeer, CEO, Genzyme Corporation.
Dr. Arthur Levinson, CEO, Genentech, Inc.
Hendrick Verfaillie, President and COO, Monsanto Company.
Dr. Lewis Edelheit, Senior Vice President, General Electric Corporate R&D.
Dr. Christine Cassel, Dept. Chair, Geriatrics and Adult Development, Mt. Sinai Medical Ctr.

Panel 3

Peter Lynch, Vice Chairman, Fidelity Management and Research Company.
Matthew Andresen, President, Island ECN, Inc.
Dr. M. Kathy Behrens, Member of the Board of Directors, National Venture Capital Association.
James Glassman, DeWitt-Wallace Reader's Digest Fellow, American Enterprise Institute.
Dr. Daniel Callahan, Co-founder, Hastings Center.

Presentation

Dr. John Kim Niparko, Director, Division of Otology, Neurology, and Skull Base Surgery, Johns Hopkins Univ School of Medicine. Accompanied by 4-year old patient Rachel Noble.

Panel 4

Edward Fritzky, CEO, Immunex Corporation.
Carl Rausch, CEO, Biopure Corporation.
Ronald Dollens, CEO, Guidant Corporation.
Dr. George Rathmann, Chairman of the Board, ICOS Corporation.
Dr. David Carbone, Director, Experimental Therapies, Vanderbilt-Ingram Cancer Center.
Dr. Joseph Smith, Chairman, Dept. of Urological Surgery, Vanderbilt University.
Dr. Alan Sager, Professor of Health Services, Boston University School of Public Health.